National Organization for Rare Disorders, Inc.®

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March 26, 1999

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Dockets Management Branch
(HFA-305)
Food & Drug Administration
5630 Fishers Lane, Room 1061
Rockville, MD 20852

Re: Docket No. 99N-0336
Talking With Stakeholders About FDA Modernization

Dear Sirs:

The National Organization for Rare Disorders (NORD) is unable to attend the April 28 meeting and teleconference to discuss the agency's progress on implementing FDAMA and seeking input from stakeholders. We are therefore submitting the following written comments in response to the *Federal Register* notice of March 22, 1999.

NORD represents an estimated 20 million Americans with rare "orphan diseases." As a representative of the patient community, we are primarily concerned about the speedy availability of safe and effective drugs, biologics, devices and medical foods. We feel the communication efforts of the FDA can be greatly improved so that the patient community will have better access to understandable information about experimental and approved therapeutics, including the risks and benefits of marketed products.

1. In response to question 1, the agency can expand its capability to incorporate state-of-the-art science into its risk-based decision making by providing more hands on training of FDA personnel. This may include laboratory research, FDA personnel's participation in the commercial production of regulated products, on-going education and training, etc. However, as part of this training FDA personnel should be required to spend some time in the clinic to learn the realities of clinical trials and sensitize them to patient needs.

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FDA is often criticized for its insensitivity to patients, especially when the agency requires placebo controlled trials for fatal diseases. While such decisions are easy to make on Fishers Lane, they are not easily complied with in a hospital (especially when children are involved). FDA personnel should be exposed to the realities of medical practice so they can understand that the agency's decisions should not be made in a vacuum, and they should always consider the human implications of their directives. In this context, we highly recommend that the agency hire at least one bioethicist who can participate in protocol design, review and amend informed consent documents, and provide oversight of clinical trials. The agency should have an internal IRB that will wrestle with ethical questions.

2. Regarding question 2, the implications of any delay in exchange of information between FDA and the patient community can be profound. We are baffled when we hear about a serious adverse effect of a marketed drug, as to why weeks or months can go by before the information is added to the drug's label. We hear that FDA is "discussing" possible label amendments with the drug sponsor, or "negotiating" label changes. If FDA's primary mission is to protect and enhance the public's health, there should be no negotiation; FDA should tell the drug sponsor to change the label. There should be no delay and no discussion if patients are at risk. FDA's public health role is compromised when the agency appears to be serving the interests of pharmaceutical or device companies rather than the interests of the public.

3. This question asks how the agency can better educate the public about the risk/benefit ratio of marketed products. We believe the first step in the process is for FDA to stop categorizing virtually every piece of information about regulated products as a "trade secret." For example, if a patient asks if there is an experimental drug for their disease, FDA is not permitted to say whether an IND has been filed because such information is a "trade secret." Nor is FDA permitted to say whether a drug is in phase II or phase III trials, nor whether an NDA or PLA has been submitted. While such information is kept secret at the agency, Wall Street investors know virtually everything about these products including where they are in the research pipeline, when the company will file an NDA, and what the results of the clinical trials have shown to date. Thus, the patient community is denied information that the investor community already knows. Patients should not have to file a *Freedom of Information* request to get information that companies have already disclosed elsewhere.

   Moreover, the FDA does not currently have the authority to require manufacturers to amend even blatantly outrageous informed consent documents, and the agency should seize this authority in order to assure that investigators and companies are communicating accurately with potential participants in human trials.

   It is also incumbent on the FDA to require all manufacturers to print understandable labeling information; package inserts and labeling printed in books such as the PDR are not helpful to people who have not received a scientific education. To deny patients understandable information about drug side effects and possible interactions is immoral and unethical, but this is the current situation for modern consumers.

4. The patient community is very sensitive to the extraordinary resource limitations of the FDA. It is imperative that sufficient resources are made available to assure that the agency can adequately carry out all responsibilities of its mandate. We are especially concerned that post-marketing surveillance activities, adverse event monitoring, generic drugs, foods, cosmetics, inspections, importation and medical device programs are suffering due to the focus of FDA resources on new drug development activities.

We suggest it was wrong for the agency not to implement user fees on new drugs until the pharmaceutical industry agreed to the fees. It is therefore wrong for the agency to avoid implementation of user fees on other industries simply because those industries do not want to pay. Public health is not enhanced by delayed availability of generic drugs, devices, etc. In other countries regulatory agencies charge user fees without the permission and consent of regulated industries. FDA should do likewise for generic drugs, devices, foods, cosmetics, veterinary drugs, etc. Congress will never give the agency sufficient resources to do everything it needs for the protection of public health, so user fees appear to be the pragmatic answer to this long-term problem.

5. Additional actions that we propose to enhance communication processes include absolute deadlines for FDA officials to answer questions from the public; it is not unusual for consumers to wait many months to receive an answer to a letter sent to the FDA. Too many times the FDA does not answer consumers' questions because the agency determines that a truthful answer is a "trade secret." FDA personnel are generally unaware that the public is a stakeholder, and the public deserves timely and accurate answers. In general, consumers do not ask questions that would violate intellectual property rights; they just want answers affecting their personal health (or the health of a loved one) that have nothing to do with patents. The agency must respond to the public appropriately for the sake of public health. For example, consumers sometimes express anger against the agency for not approving a new drug, but the agency will not freely reveal that the drug is not approved because an NDA has not been filed.

   Additionally, the agency must act on the public's behalf to require label changes (without the manufacturers consent), require doctors and hospitals to report adverse effects, require withdrawal of dangerous products (such as nutritional supplements), and in general protect the public health even when a regulated industry disagrees. There are times when consumers feel that the public is more regulated by the FDA than are the industries that the agency is supposed to be monitoring. When a member of the public is told to write a Freedom of Information letter in order to find out if a certain side effect has been reported for a specific drug, we are being regulated rather than the manufacturer of the drug.

I do hope these comments are helpful in understanding the public's frustration with the FDA. We sincerely appreciate the Commissioner's stakeholder meetings which provide an opportunity to the public to make their views known to the agency.

Very truly yours,

Abbey S. Meyers
President

ASM:aa

cc: Sharon Smith Holston
Deputy Commissioner for External Affairs

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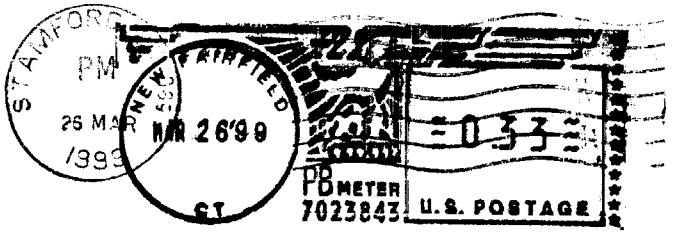

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